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On Behalf of the
BIOTECHNOLOGY INDUSTRY ORGANIZATION (BIO)

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Good morning, Mr. Chairman and Members of the Committee. My name is Dr. Charles Johnson. I am Associate Director of Specialty Biotherapeutics at Genentech, Inc., a leading biotechnology company headquartered in South San Francisco, California. I am here today representing the Biotechnology Industry Organization (BIO). BIO represents more than 1000 biotechnology companies, academic institutions and state biotechnology centers in all 50 U.S. states and 33 other nations. BIO's members are involved in the research and development of medical, agricultural, industrial and environmental biotechnology products.

Most of the hard work in our industry is directed toward research on currently unmet medical needs: new therapies and cures for various cancers, Alzheimer's and Parkinson's diseases, diabetes, heart disease and hundreds of other debilitating and life-threatening illnesses.

Thank you, Mr. Chairman, for holding this hearing on such an important issue: how to effectively protect those who voluntarily participate in our research while, at the same time, facilitating critical medical research. As you and your colleagues examine this issue, I urge you to remember two critical facts.

First, participants in research are volunteers, meaning that we must do all we can to ensure that they have the utmost confidence that they will be protected.

Second, medical research has and will continue to lead to cures and treatments for millions of Americans suffering from diseases. One hundred seventeen biotechnology products have helped a quarter billion people worldwide thus far, and another 350 biotech medicines targeting more than 250 diseases are in late stage development. Many of these are diseases that are currently incurable.

Much attention has been given lately to issues surrounding the protection of the volunteers who participate in our research. As you are already aware, Mr. Chairman, medical research is a heavily regulated activity – our products and manufacturing processes are regulated by the Food and Drug Administration (FDA), and our research protocols are reviewed and scrutinized by Institutional Review Boards (IRBs) under an extensive set of federal regulations governing research (the federal Common Rule). Moreover, virtually all states have developed regulations that affect research. In addition, the HIPAA privacy rule imposes a new layer of review and oversight over our research.

Despite this extensive regulation, some have called for additional restrictions to be instituted relating to consent, IRB accreditation and review, and conflicts of interest.

From many different perspectives, reform of the existing system is not only necessary and desirable, but appears inevitable. In light of this, BIO companies have spent considerable time evaluating the existing system of research oversight. Based on this analysis, we have identified

several key concerns and areas for improvement. They are:

- Multiple and overlapping layers of review, leading to confusion and inefficiency for participants as well as research sponsors;
- New regulations that will increase the burden on an already overwhelmed IRB system;
- An existing framework for review of research involving human participants that is inappropriate for research involving medical archives or data;
- Differing state laws govern and complicate the form of research review and format of consent required in each state; and
- A strong and persistent perception that the presence of private money in the health care setting creates conflicts of interest in researchers that may affect results and the quality of care provided to research participants.

Multiple Layers of Review

The current system of research review relies heavily on IRBs. Historically, they have filled the important role of providing independent review of research projects. However, the current regulatory system applies multiple overlapping layers of review for sponsors of every clinical protocol. Specifically, FDA regulations require the sponsor to obtain review by an IRB, and each investigator affiliated with an academic institution must have its IRB separately review and approve every aspect of the research protocol under federal regulations that apply to institutions that receive federal grant money. Consequently, trials that take place in

several locations must be reviewed by several different review bodies. Each can require changes to trial design, the informed consent form, or any other protocol component. This adds enormous complexity and expense to a research project.

An additional complication is the HIPAA privacy regulation governing the use and disclosure of medical information. That regulation adds an entirely new authorization process to the informed consent already required from every research participant and/or data subject. It requires that researchers get an individual's authorization – or a waiver of authorization from an IRB or privacy board – to access and use protected health information for research purposes. The IRB's review of this issue is in addition to its consideration of the other risks present to research participants.

Thus, two distinct assents are now required of each research subject: informed consent to participate in research *and* "authorization" to disclose and use an individual's protected health information in research under the HIPAA privacy regulation.

As to the overall issue of the growing multiple layers of review, BIO believes Congress should eliminate the multiple separate legal reviews currently required for clearance of a sponsored clinical research protocol. Mechanisms should be developed to centralize and streamline review of research projects. In addition, researchers should be allowed to use patient information without authorization where researchers (1) secure individuals' informed consent or (2) obtain a waiver of consent by an IRB or

privacy board, in whole or in part, where waiver is warranted under existing law. In addition, we support modifying the criteria for waiver of consent/authorization for use of patient data and archival information both in the privacy rule and under the current Common Rule to enhance access to much-needed data where the confidentiality risks present to the individual are minimal.

In this regard, we note that HHS recently proposed modifications to the HIPAA privacy rule that would simplify and streamline the requirements for authorization by IRBs and privacy boards. BIO supports these proposed changes as an important first step in eliminating unnecessary and inappropriate regulatory hurdles for the conduct of research, and we urge HHS to adopt these modifications in its revised final rule. Without these changes, the existing waiver of authorization standard, in particular, is unworkable and will have a significant adverse impact on research activities.

In addition, since IRBs play such an important role in the research oversight system, BIO believes they should be held accountable for meeting their responsibilities. Some have recommended that a system of accreditation for IRBs be developed. BIO is intrigued by the concept of IRB accreditation and would be supportive of exploring the issues involved.

Review Commensurate with Risk

Currently, research studies are reviewed using the same criteria regardless of the type of risk faced by the research participant. For

example, a research study that entailed testing a drug on individuals will be regulated the same way as a study that relied only on a review of medical records. This process does not acknowledge the different types of risk faced by the research subjects in each study. Participants in the first study will confront safety risks, while subjects in the second study face risks related almost entirely to confidentiality.

The regulatory structure stems from the history of our oversight system that based federal review on factors other than the risk to the research participant, such as presence of federal funding or regulation. BIO believes that this paradigm is no longer appropriate – for researchers or research participants. As we learn more about how genomic information can be used to cure disease, medical records review and archival research will grow in importance.

Thus, BIO supports an alternative approach that makes regulatory oversight commensurate with the risk to the research participant. That type of system would establish one set of requirements for research that involves intervention or interaction with individual research participants and a separate set of requirements tailored to the unique issues raised by research using medical records and tissue archives. This new framework would be applicable to all research, regardless of its funding source. It is important to note that in a report issued last year, the National Bioethics Advisory Commission (NBAC) made a similar observation, and endorsed the notion that review should be commensurate with the types of risk presented by the research.

Differing State Laws

A related problem is that researchers are subject to a patchwork of different, and sometimes inconsistent, state laws. Although there are extensive federal rules regarding research, state laws govern issues such as the form of review and format of additional documentation of consent.

This is often problematic for researchers. For example, new state laws pertaining to genetic analysis are quite restrictive, requiring additional separate consents and imposing onerous requirements regarding the use and retention of tissue and blood samples that sometimes are inconsistent with FDA requirements.

A 1999 study of state health privacy laws showed the vast differences among the states. In addition to existing differences, state laws in this area are in flux. During the 2000 state legislative session, 26 states debated laws concerning privacy. This turbulent environment will slow important research efforts.

It is important to note that the differences among states do not seem to start from differences in the level or degree of protection, but reflect different state legislatures' views of the specific procedures or requirements for accomplishing the same objective. Nonetheless, the requirements and penalties are different enough to require every researcher to hire lawyers to assure compliance with the laws of more than 50 states and local jurisdictions in designing informed consent documents for a multi-state trial.

To remedy this problem, BIO believes that consideration should be given to creating one national, uniform set of rules governing research. National standards would allow researchers to create informed consent and other procedures that will be legal in all states. These federal research standards should preempt state laws that create conflicting obligations regarding research participants from different states.

Conflicts of Interest

There is a strong and persistent perception that the presence of private money in the health care setting creates conflicts of interest in researchers that may affect results and/or the quality of care provided to research participants. This perception has the potential to damage the public's trust in biomedical research.

We must take steps to maintain public confidence. However, it is important to remember that the tremendous investment by the private sector over the past two decades has led to remarkable medical breakthroughs. Government policy to encourage private investment has been a major factor in the development of a biotechnology industry in the United States that is the envy of the world.

The best ways to both protect patients and the integrity of research is to ensure that research protocols are independently reviewed and that all financial interests are disclosed. We understand that the academic institutions are in the process of carefully reviewing conflict of interest

issues and are attempting to generate a unified position and set of policies regarding financial interests. In the meantime, BIO agrees with the direction of the NBAC recommendations, which is to focus the discussion in a way that encourages disclosure of financial relationships between and among researchers, investigators and IRBs, but does not prohibit nor otherwise impose rigid restrictions on the existence of such relationships.

Conclusion

Mr. Chairman, we believe that it is appropriate to review the existing regulatory structure for research and urge that consideration be given to BIO's four key principles: (1) eliminate multiple separate levels of review; (2) modify the regulatory framework so that review is commensurate with the type of risk involved for the research participants; (3) preempt state laws that create conflicting obligations; and (4) work with academic medical centers and other affected entities and individuals to develop an approach for addressing real and perceived conflicts of interest.

BIO companies believe that it is critical to make sure that, despite the changes in our research infrastructure over the years, participants continue to be protected. We firmly believe that addressing these key issues described above will enhance the level of protections we can guarantee participants in our research projects.

In protecting our research participants, we must also ensure the continuation of valuable – potentially life-saving – research. We are fortunate to live in an era of enormous promise as scientists begin to

access a vast library of genetic information with the goal of improving our medical interventions. Decades of responsible science have shown that protecting research participants and promoting medical research are mutually attainable.

BIO looks forward to working with the Committee as it pursues both goals.

Thank you.